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Compliance of comprehensive care in children with Duchenne muscular dystrophy: Real-world experience of a pediatric neuromuscular diseases center

Duchenne musküler distrofili çocuklarda kapsamlı bakıma uyum: Bir pediatrik nöromüsküler hastalıklar merkezinin gerçek dünya deneyimi

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ABSTRACT

BACKGROUND

Although international care considerations for Duchenne muscular dystrophy (DMD) were revised in 2018, compliance in clinical practice remains unknown. The objective of this study was to investigate the parental compliance with care considerations in a tertiary hospital in Türkiye.

METHODS

A questionnaire was administered to parents of patients with DMD to assess compliance with recommended frequencies of visits to healthcare providers and clinical tests. The data collected from the questionnaires were analyzed using cross-tables to explore if there were differences between ambulant and non-ambulant patients.

RESULTS

Sixty-one participants completed the questionnaire, most of whom were parents of ambulant patients (n= 44, 72.1%). The majority of parents complied with the recommended frequency of cardiac assessments. However, suboptimal compliance was observed in the rest of the assessments, including the PPSV23 and influenza vaccination rates, which were notably low across all patient groups. Compliance with anthropometric assessments and the use of corticosteroids with proper posology was significantly higher in ambulant patients. Compliance with respiratory and orthopedic evaluations was significantly higher among non-ambulant patients.

CONCLUSION

This study showed several areas in which compliance is insufficient. To improve compliance, future work should focus on home-based assessments, educational programs for patients, parents, and physicians, as well as raising awareness about the importance and timing of recommended vaccinations

KEYWORDS

Compliance, Duchenne muscular dystrophy, delivery of health care, guideline,

ÖZ

AMAÇ

Duchenne Musküler Distrofisi (DMD) için uluslararası bakım önerileri 2018 yılında güncellenmiştir, ancak klinik uygulamalardaki uyum bilinmemektedir. Bu çalışmanın amacı, Türkiye'deki bir üçüncü basamak hastanede ebeveynlerin bakım önerilerine uyumunu araştırmaktır.

GEREÇ YÖNTEM

DMD'li çocukların ebeveynlerine, bakım önerilerinden elde edilen önerilen sıklıklara göre sağlık hizmeti sağlayıcı ziyaretleri ve klinik testlere uyumlarını değerlendiren bir anket uygulanmıştır. Anketlerden elde edilen veriler, ambulatuvar ve non-ambulatuvar hastalar arasında fark olup olmadığını incelemek amacıyla çapraz tablolar kullanılarak analiz edilmiştir.

BULGULAR

Ankete 61 ebeveyn katılmış olup, katılımcıların çoğunluğu ambulatuvar hastaların ebeveynlerinden oluşmaktaydı (n = 44, %72,1). Çoğu ebeveyn, kardiyak değerlendirmeler için önerilen sıklıklara uyum göstermiştir. Ancak PPSV23 ve influenza aşı oranları dahil diğer değerlendirmelerde optimal düzeyde uyum sağlanamamıştır. Antropometrik değerlendirmeye uyum ve kortikosteroidlerin uygun dozda kullanımı ambulatuvar hastalarda anlamlı olarak daha yüksek bulunmuştur. Solunum ve ortopedik değerlendirmelere uyum ise non-ambulatuvar hastalarda anlamlı olarak daha yüksektir.

SONUÇ

Bu çalışma, bakım önerilerine uyumun yetersiz olduğu çeşitli alanları ortaya koymuştur. Uyumun artırılması için evde yapılabilecek değerlendirmeler, hastalar, ebeveynler ve hekimler için eğitim programları ve önerilen aşıların önemi ve zamanlaması konusunda farkındalığın artırılması üzerinde durulmalıdır.

ANAHTAR KELİMELER

Duchenne musküler distrofisi, kılavuz, sağlık hizmetleri aktarımı, uyum

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Pathogenic changes in the dystrophin-encoding DMD gene gives rise to life-threatening Duchenne muscular dystrophy (DMD), which is the most common muscular dystrophy with an incidence of approximately 1:5000 to 1:20000 in male live births (1). The variable but usually severe clinical course makes the multidisciplinary approach and strict follow-up indispensable. Experience and disease-specific clinical approach are essential for best-practice management of rare diseases including DMD. However, a lack of knowledge regarding the recognition of multisystem involvement and complications may cause delayed guidance and suboptimal management for the patients, leading to a worse health status and subsequently lower levels of participation and quality of life (2). Recently, a considerable literature has grown up around the theme of addressing the needs of patients whose survival time is quite prolonged by the efforts of multidisciplinary teams led by neuromuscular specialists. However, disruptions in the comprehensive care for individuals with DMD continue to ocur (3–5). This study seeks to assess the compliance of the parents of individuals with DMD regarding follow-up recommendations and to what extent the care complies with the international care considerations.

Materials and Methods

The present study was conducted on patients who were aged between 0 and 20 years old with DMD, who attended pediatric neurology department of Dokuz Eylül University Faculty Of Medicine during a period of 10 years between 2008 and 2017. Telephone interviews were conducted to assess parental compliance with internationally recommended care considerations. During the calls, parents were informed about the purpose of the study, and verbal consent was obtained. Demographic data were also recorded. The interviews included structured, closed-ended questions that focused on whether families adhered to the recommended follow-up schedule in various domains, including neuromuscular, respiratory, cardiac, and bone health assessments, physical rehabilitation, endocrine and orthopedic evaluations, swallowing-gastrointestinal management, steroid treatment, immunization, psychosocial functions, and genetic counseling. For each domain, parents were specifically asked whether the recommended evaluations or interventions had been performed at the suggested frequency based on international care guidelines. Compliance was categorized as either 'performed at recommended frequency' or 'not performed at recommended frequency'. The recommended frequency for assessingeach item was determined based on care considerations (3–5).

Evaluation of muscle strength, range of motion, and motor function tests at least twice per year, as well as twice per year visits to a neuromuscular specialist were considered optimal care for neuromuscular management (3). The outcome measures for cardiac management included a baseline evaluation at the time of diagnosis and an annual visit with a pediatric cardiologist. Baseline cardiac evaluation consisted of past and present cardiac medical history, family history, physical examination, electrocardiogram and non-invasive imaging techniques (echocardiogram for <6-7 years old children, cardiac magnetic resonance imaging for ≥6-7 years old children). Annual cardiological assessment included cardiac medical history, physical examination, electrocardiogram and non-invasive imaging techniques. The optimal assessment frequency for patients with cardiac symptoms was determined based on a recommendation of a pediatric cardiologist. Respiratory management consisted of annual and bi-annual spirometric evaluations for ambulant and non-ambulant patients, respectively (4). Orthopedic management consisted of a visit to an orthopedic surgeon and visual inspection of spine annually and bi-annually for ambulant and non-ambulant patients, respectively (4). Optimal gastrointestinal evaluations included twice-yearly assessments of dysphagia, constipation, gastroesophageal reflux, and gastroparesis. Since the recommended care considerations of nutritional and anthropometric profile assessments are covered by both gastroenterological and endocrinological evaluations, these particular items were analyzed among subgroups of endocrinological evaluations to avoid confusion. Outcome measures for endocrinological management consisted of screening for overweight, growth retardation, delayed puberty, and bone demineralization. Optimal anthropometric evaluations consisted of weight, height, and body mass index measurements were taken twice a year. The optimal frequency of puberty assessment using Tanner staging was performed every six months by the age of nine.

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Monitoring of bone health included annual assessments of dietary calcium intake and serum 25-hydroxy-vitamin D concentration (3). For the item of psychosocial assessment, due to the lack of a certain recommended frequency in 2018 guideline (5) except for at the time around diagnosis, before school entry and after a psychological function change, optimal compliance was evaluated according to whether or not they were screened during the aforementioned periods and whether or not they followed the recommendation to refer the patients to psychiatrists, psychologists, and therapeutic educators for psychological and neuropsychological assessments and interventions when necessary. Besides the National Immunization Program (NIP) of Türkiye (Table 1), (6) the pneumococcal polysaccharide vaccine 23 (PPSV23) and the annual inactivated influenza vaccines were questioned. For the item of genetic counseling, families were specifically asked whether they had received formal counseling from certified genetic specialists. While neurologists may provide basic genetic information, our study focused on evaluating compliance with formal genetic counseling recommendations.

Table 1. Turkish ministry of health national immunization program

Vaccines	Birth	End of 1 month	End of 2 months	End of 4 months	End of 6 months	End of 12 months	End of 18 months	End of 24 months	Elementary 1 degree	Elementary 8 degree
HepB	I	II			III					
BCG			I							
PCV			I	II		B				
DaPT-IPV-Hib			I	II	III		B			
OPV					I		II			
VAR						I				
MMR						I			II	
HepA							I	II		
DaPT-IPV									B	
Td										B

HepB, Hepatitis B Vaccine; BCG, Bacille Calmette-Guérin (Tuberculosis) Vaccine; PCV, Pneumococcal Conjugate Vaccination; OPV, Oral Polio Vaccine; VAR, Varicella vaccine; MMR, Measles, Mumps & Rubella Vaccine; HepA,Hepatitis A Vaccine; DaPT-IPV, Diphtheria and tetanus toxoids and acellular pertussis adsorbed and inactivated poliovirus vaccine;
Td,Tetanus and diphtheria toxoids adsorbed; B, Booster.

The data from the questionnaires were validated with hospital electronic system and archival records. Patients with data incongruity between interview results and archive records were not included. The data collected from the questionnaire were evaluated in terms of significant difference between patients with/without corticosteroid therapy, and ambulant/non-ambulant patients. These subgroup variables were selected because ambulation status reflects a major milestone in the disease course, directly influencing both accessibility to hospital visits, and parental psychological responses, whereas steroid therapy is the cornerstone of treatment in DMD, usually discontinued after loss of ambulation. In addition, parental concerns about steroid-related side effects may particularly affect their compliance with vaccination and preventive care, making it a relevant variable for analysis.

Ethical approval

The present study was conducted in accordance with the 1964 Declaration of Helsinki and approved by the Ethics Committee of Dokuz Eylül University Hospital (number of approval: 2017/21-56).

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Statistical analysis

All statistical analyses were conducted using Statistical Package for Social Sciences (SPSS) version 24.0 software for Windows (IBM Corp, Armonk, New York). The variables were investigated using visual (histograms and probability plots) and analytical methods (Kolmogorov–Smirnov test) to determine whether they were normally distributed. While continuous variables were expressed as mean ± standard deviation (SD) and median with minimum-maximum, categorical variables were expressed as a number and percentage. The chi-square and Fisher's exact tests were used to evaluate differences in categorical variables. A p value <0.05 was established as the threshold for determining statistical significance.

Results

Demographic features

A total of 78 patients were evaluated for a diagnosis of DMD, of whom 6 were excluded due to lack of telephone accessibility and 10 were excluded due to failure to obtain consent. One patient was further excluded because of the data inconsistency between the interview results and the electronic archive records. A total of 61 patients were enrolled, all of whose parents agreed to participate in the study. The cohort consisted entirely of male patients, with a median age of 106 (8-240) months. The majority of the cohort was ambulant (n= 44, 72.1%).

Compliance of the parents of the ambulant and non-ambulant patients

Fifty-seven percent (n= 34) of the interviewees reported regularly neuromuscular clinical visits, muscle strength assessment, and validated range of motion and motor function tests. Although compliance with neuromuscular follow-ups was more common in ambulant patients (n = 26, 29.1%), no significant difference was obtained (p = 0.396). Home-based and inpatient physical rehabilitations were received by 42 (68.8%) and 39 (64%) patients, respectively. There were no significant differences between ambulant and non-ambulant patients in either physical rehabilitation setting. While an orthopedic evaluation was not regularly performed for even half of the ambulant group, the majority of non-ambulant patients (n = 14, 82.3%) were regularly assessed in this particular respect, resulting in a significant difference between the two subgroups (p = 0.014). Overall, 57.4% (n = 35) of the cohort complied with orthopedic care considerations.

The majority of those who responded to the item about cardiological evaluations, regular electrocardiographic and echocardiographic examination rates were relatively high, with 52 patients each (85.2%). Neither of ambulant and non-ambulant patients demonstrated significant difference in electrocardiography (p = 0.692) and echocardiography (p = 0.682) rates. Cardiac magnetic resonance imaging was not performed in any of the patients. The relatively high rate of cardiac evaluations was not valid for the recommended visit frequency for a respiratory clinical assessment with spirometric examination, which was complied by only 15 (24.6%) patients. Subgroup analysis revealed that just over half of the non-ambulant patients (n = 9, 53%) were followed up with regular respiratory clinical assessments that included spirometric examinations, while only 13.6% (n = 6) of the patients complied with respiratory care considerations, resulting a significant difference between these subgroups (p = 0.001).

Detailed gastroenterological evaluations including assessments of dysphagia, constipation, gastroesophageal reflux, and gastroparesis were performed regularly in 30 (49.2%) patients without any significant difference between ambulant and non-ambulant patients (p = 0.132). Moreover, in 47.5% (n = 29) of the cohort, a complete endocrinological evaluation encompassing preventive strategies against impaired bone health, growth, and puberty was conducted. In the first part of the endocrinological evaluation, participants were asked whether they acted on the recommendation of monitoring anthropometric profiles. Of whom, 77% (n = 47) were followed up on a regular basis and the difference between the ambulant (n = 37, 84.1%) and non-ambulant (n = 10, 58.8%) groups was significant (p = 0.035). However, assessments of puberty and bone health were much lower in both ambulant and non-ambulant patients without any significant difference (p = 0.078). In 28 (46%) patients, psychosocial assessment was found to be optimal in align to care considerations.

The entire cohort was vaccinated according to the NIP of Türkiye which includes the pneumococcal conjugate vaccine (PCV13), but not the PPSV23. All patients were given a primary series of PCV13, at ages 2 and 4 months with a booster at age 12 months. However, the influenza and PPSV23 vaccines were

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equally low in the cohort (n = 17, 27.9%) and its subgroups (ambulant; n = 15, 34.1% and non-ambulant; n = 2, 11.7%), revealing no significant difference (p = 0.081). Table 2 presents the results obtained from the statistical analysis of ambulant and non-ambulant patients.

Table 2. Compliance rates of the parents of ambulant and non-ambulant patients with Duchenne muscular dystrophy

	Ambulant (n= 44) *n, %*	Non-ambulant (n= 17) *n, %*	*P*
Neuromuscular evaluation			
Neuromuscular specialist visit	26 (59.1%)	8 (47%)	0.396
Muscle strength assessment	26 (59.1%)	8 (47%)	0.396
Range of motion assessment	26 (59.1%)	8 (47%)	0.396
Motor function tests	26 (59.1%)	8 (47%)	0.396
Physical rehabilitation (home-based)	32 (72.2%)	10 (58.8%)	0.293
Physical rehabilitation (inpatient)	30 (68.2%)	9 (53%)	0.266
Cardiac evaluation			
Baseline evaluation at diagnosis	44 (100%)	17 (100%)	ND
Electrocardiography	38 (86.4%)	14 (82.3%)	0.692
Echocardiography	37 (84.1%)	15 (88%)	0.682
Cardiac MRI	0	0	ND
Respiratory evaluation – spirometry	6 (13.6%)	9 (53%)	**0.001**
Orthopedic evaluation	21 (47.7%)	14 (82.3%)	**0.014**
Swallowing and gastrointestinal evaluation	19 (43.2%)	11 (64.7%)	0.132
Endocrinological evaluation			
Growth (height)	37 (84.1%)	10 (58.8%)	**0.035**
Weight	37 (84.1%)	10 (58.8%)	**0.035**
Bone health	24 (54.5%)	5 (29%)	0.078
Puberty	24 (54.5%)	5 (29%)	0.078
Psychosocial evaluation	19 (43.2%)	9 (53%)	0.493
Routine immunization schedule	44 (100%)	17 (100%)	ND
Influenza vaccine	15 (34.1%)	2 (11.7%)	0.081
Pneumococcal vaccine (PPSV23)	15 (34.1%)	2 (11.7%)	0.081
Genetic counselling	40 (91%)	15 (88%)	0.753
Corticosteroid therapy	24 (54.5%)	5 (29.4%)	0.078
Prednisolone	14 (32%)	1 (5.8%)	**0.035**
Deflazacort	10 (22.8%)	4 (23.5%)	0.947
Proper posology	23 (52.3%)	4 (23.5%)	**0.043**

*: Patients were evaluated with one or more of the motor function tests including six-minute walk test, North Star Ambulatory Evaluation, Brooke Upper Extremity Scale, and Vignos Lower Extremity Scale.
PPSV23, pneumococcal polysaccharide vaccine 23;ND, no difference sufficient for statistical analysis; MRI, magnetic resonance imaging; A p-value < 0.05 was considered as significant.

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Compliance of the parents of the patients with and without corticosteroid therapy

Twenty-nine (47.5%) patients were receiving corticosteroid therapy, in whom most of them were ambulant (n = 24, 54.5%). There was no significant difference in steroid use between ambulant and non-ambulant patients (p = 0.078). The median age of corticosteroid therapy initiation was 4.2 (4–5 years) years. Prednisolone use was significantly higher among ambulant patients (n = 14, 32%, p = 0.035). Deflazacort, however, was not significantly associated with the motor function classification of the patients (p = 0.947). Among patients receiving corticosteroid therapy, the use of guideline-recommended dosages (0.75 mg/kg/day for prednisolone and 0.9 mg/kg/day for deflazacort) were significantly more common in ambulant patients compared to non-ambulant patients (52.3% vs. 23.5%, p = 0.043). (Table 1).

Influenza vaccination rates were low in both the corticosteroid group (n= 8, 27.6%) and the no corticosteroid (n= 9, 29%) group withno significant difference between the two groups (p= 0.901). A similar result was observed for the pneumococcal vaccination rates between the two groups (p= 0.225); 7 (24.1%) and 12 (38.7%) patients received pneumococcal vaccine in the corticosteroid and no corticosteroid groups, respectively. Figure 1 provides the results obtained from chi-square analysis of vaccination status of the patients depending on wheter they received or not reeived corticosteroid therapy.

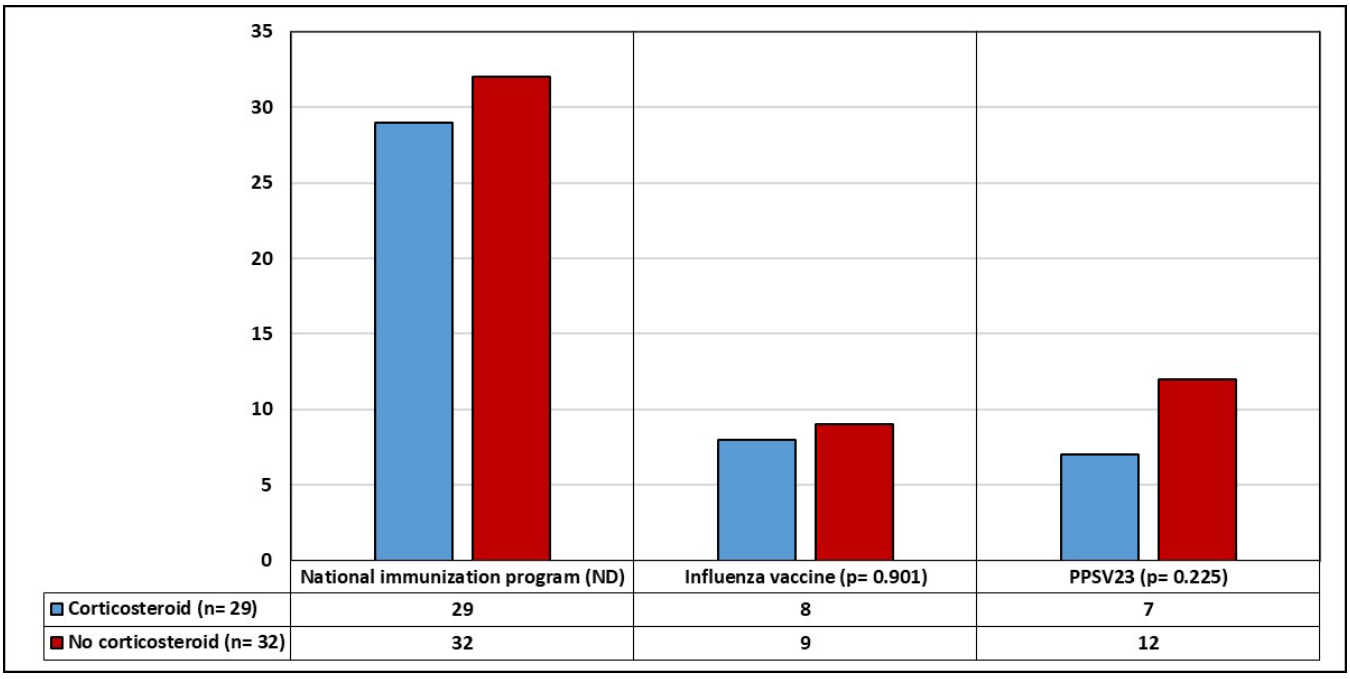


Figure 1. Bar graph showing vaccination status of individuals with Duchenne muscular dystrophy with and without corticosteroid therapy. ND, No difference sufficient for statistical analysis; PPSV23: Pneumococcal polysaccharide vaccine 23

Discussion

The present study revealed that the real-world experience of parental compliance among individuals with DMD in Türkiye falls short of the standards specified in international care considerations in many aspects. In which, the crucial role of regular visits to a neuromuscular specialist and the indispensability of various neuromuscular assessments, such as muscle strength, range of motion, and motor functional tests were highlighted (3). In our cohort, just over half of the participants met the recommendation to visit a neuromuscular specialist twice a year. Despite the results in a wide spectrum of 27-86.9% reported from different countries, (7–10). it is clear that more comprehensive education is needed

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to ensure the parental compliance to these visits at the recommended frequency. Of note, in the present cohort, the frequency of neuromuscular assessments was the same with that of visit a neuromuscular specialist who performed these evaluations. In fact, rates of neuromuscular assessments were consistent with that of motor function tests ranging between 23 and 53.8% and range of motion assessments ranging from 31.9 and 64% in the literatüre (7,9). Despite no significant difference between ambulant and non-ambulant patients, lower compliance with neuromuscular evaluation in non-ambulant patients corresponds to suboptimal results in other studies (7,9,10) This result may be explained by the challenges in transportation of non-ambulant patients to the hospital as well as difficult transfer from a wheelchair to a treatment table, where these assessments are performed. The assessment methods should be revised so that these particular tests can also be performed while sitting on a wheelchair, especially in late-stage non-ambulant patients. More effort is therefore needed to increase parental compliance, since regular monitoring of function decline and prominence of contractures is essential for early interventions that have a major impact on mortality and morbidity.

In a larger-cohort study of patients from Germany, Italy, UK, and USA, insufficient compliance with cardiological evaluation recommendations was demonstrated, ranging between 34 and 66% (10). Nevertheless, there are a few areas in our study where practice was aligned with international care considerations about cardiac assessments, including baseline cardiological evaluation. Moreover, despite no significant difference between ambulant and non-ambulant patients, the rate of performing electrocardiographic and echocardiographic examinations at the recommended frequency was high, consistent with the recent literatüre (7,9) The observed increase in compliance rates in recent studies could be attributed to higher awareness of parents and patients, as well as easier accessibility to cardiac specialists over time.

In contrast to earlier findings with relatively high compliance rates of respiratory care considerations, (7,9,10) no evidence of sufficient follow-up for this particular item was detected in the present study. Moreover, in our study, poor compliance was significantly higher in ambulant patients, which is not in agreement with those obtained by Heutinck et al., (7) who reported a higher compliance in ambulant group.

The authors speculated that all recommended tests were no longer clinically relevant and might only cause burden on the non-ambulant individuals requiring non-invasive ventilation (7). On the other hand, several reports have shown similar proportions of compliance for both ambulant and non-ambulant patients (10–12). Our results displayed the expected pattern of increased compliance with care considerations in advanced disease stages, where there is growing concern about prognosis for patients and parents. Similarly, significantly higher rate in non-ambulant patients was valid for orthopedic evaluation with higher compliance rates in both groups. In fact, the rate observed in this study (57.4%) is far above the reported range of 18.5 and 40% in several studies (7,9,10). Although the rates of respiratory and orthopedic evaluations are higher than those reported in the literature, regular monitoring of both functions is imperative for early intervention. Therefore, these rates should be increased even more.

Swallowing dysfunction (dysphagia), which usually begins in the teenage years, is universal and progressive in patients with DMD, leading to weight loss, dehydration, episodes of suffocations, and aspirations (3,13) Although underlined in care considerations, very little attention has been paid to obtain data about constipation, gastroesophageal reflux, and gastroparesis in DMD patients, which are all assumed to be highly prevalent, underrecognized, and undertreated (3,14,15).Optimal swallowing and gastroenterological assessment at recommended frequency was achieved in only half of the cohort. This result highlights the fact that both physicians and parents should pay more attention to this particular issue in order to prevent further complications and increase the quality of life.

Due to multifactorial etiology, particularly corticosteroids, disruptions in growth, bone health and puberty are common in DMD patients (3,16,17) However, limited data on compliance with these care considerations have been published (7,9). Compliance with the recommended frequency of weight and height measurements was reported between 50.7and 63% and 29.2 and 57%, respectively (7,9). Our study with a compliance rate of 77% suggests that attention of the parents to this item has increased over time, although the frequency at which more intense focus is needed is still

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insufficient. Nonetheless, compliance with evaluation of anthropometric profile was not specified separately in ambulant and non-ambulant patients, which was found at a significantly higher rate in ambulant patients in the present study, which could be partly attributed to difficulty of these measurements secondary due to the immobility. Another significant poor compliance was detected in assessments of bone health with a rate of 47.5% in our study, consistent with reported rates ranging from 29.5% to 68% (7,9).

Although the psychologically distressing effect of delayed puberty is stated in care considerations, (3) there remains a paucity of studies evaluating compliance of patients and/or parents with this particular issue. Although evaluations of bone health and puberty at the recommended frequency were higher in ambulant patients, these differences were not statistically significant in the present study, necessitating further works with larger cohorts and education for both parents and physicians, as corticosteroids remain the core therapy.

Along with longer survival in DMD patients who now live in their thirties and beyond, a whole new set of concern topics about mental health and psychosocial care requires to be brought on the agenda (18,19. However, the rate of compliance with psychosocial assessments remained low, with reported compliance rates varying between 15-64%. In our cohort, less than 50% of patients complied with an optimal psychosocial assessment, indicating that increased effort must be devoted to further integrate psychosocial management throughout entire disease course.

In our study, we assessed whether families had accessed specialty services, but we did not systematically evaluate how effectively these follow-ups were maintained. It is important to note that obtaining timely appointments with relevant specialties and subspecialties can be challenging due to health system limitations. These difficulties may contribute to reduced adherence to international care recommendations, as families may encounter barriers in securing regular follow-up visits. Although our findings suggest that such systemic challenges could negatively affect compliance, this remains a possible explanation rather than a directly measured outcome in our cohort.

Corticosteroids are the core standard therapy for patients with DMD based on evidence that they improve muscle strength and motor function, delay loss of ambulation, improve pulmonary function, reduce the need for scoliosis surgery, delay onset of cardiomyopathy, and overall leading to longer survival (20). However, corticosteroid use differed substantially in several recent studies, ranging between 46.4and 73%. (7–9,21). Despite vigorous efforts and education, the rate of past or current corticosteroid status was less than 50% in our cohort.

The low rates are thought to be largely due to concerns about possible side effects (8,21). Even though care considerations clearly and unequivocally emphasize the indispensable role of corticosteroids, concerns regarding the side effects of daily regimens have led to several alternative dosing schedules with lower/higher doses or intermittent pattern (21). Over time, initial posology and titration protocols have been established for deflazacort and prednisolone/prednisone (3). However, in the present study, inappropriate posology was common probably due to limited compliance with medical treatment, fear of side effects, or poor compliance with weight management. The fact that the proper posology was more common in ambulant patients can be explained by better compliance of these patients with neuromuscular management and anthropometric measurements.

Under the NIP of Türkiye, vaccines provide protection against 13 different infectious agents with 10 sessions in a total of 21 individual vaccinations (6] Although the PCV13, one of the vaccines included in the NIP, was given to each patient according to the schedule recommended by CDC and care consideration (5,22), the PPSV23 vaccine failed to be administered in the recommended schedule. At least eight weeks following completion of PCV13 vaccination, these patients were recommended to get the first of two doses of PPSV23, spaced five years apart (5,22). According to this schedule, the PPSV23 vaccination rate was only 27.9%, which was also valid for the inactivated influenza vaccine. A possible explanation for the very low vaccination rates compared to 75and 91.6% in current studies (7,9). is that the PPSV23 and influenza vaccines are expensive, unlike free of charge vaccines included in the NIP. This possible explanation is also supported by the fact of no significant difference between the influenza and PPSV23 vaccines in patients with and without corticosteroid therapy; the rates of these vaccines were almost

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equally low in both groups, whereas vaccines in NIP were consummate.

These results suggested that the low vaccination rates may be due to economic reasons rather than corticosteroid-vaccine interaction concerns. Although economic concerns were frequently mentioned informally by caregivers during clinical encounters, the survey did not include a structured item addressing this issue. Therefore, this interpretation should be considered preliminary. Additionally, it is highly likely that insufficient awareness among caregivers and even some healthcare providers about the importance and timing of vaccines such as PPSV23 and the influenza vaccine for patients with DMD also contributes significantly to the low vaccination rates observed in our study. Further work-up should be undertaken to improve vaccination rates by increasing the availability of these vaccinations and efforts to reduce possible prejudices against vaccines.

Overall, poor compliance with many assessments can be explained by the large number of assessments recommended based on care considerations, and it may not be feasible for parents to implement all of these assessments. However, aside from giving up these frequencies, even more frequent evaluations may be required in personalized programs. Besides, as the disease progresses, the increase in compliance with cardiac, respiratory and gastroenterological evaluations may have led to the neglect of evaluations in other areas. Developing home-based care strategies for patients who fail frequent hospital visits may be the solution for optimal care.

Conclusion

We demonstrated that iseveral areas of care considerations need improvement. However, there are a few areas in which practice aligns well with recommendations. We recommend increasing efforts even further to educate patients and parents in detail about the incontrovertible benefits of full compliance with care considerations.

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Conflict of Interest
None.

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